SBIR Grant Program

Development of OBI-1 (recombinant porcine FVIII)

Who Develops Products for Rare Disorders?

	"Big Pharma"	Start-up company
Ideal target objective	"Blockbuster", "First in Class", >$1 billion	Profitable product; worthwhile return on investment (ROI)
Ownership	Publicly owned, R & D budget	Privately owned, angel investors
In-house depth, breadth of personnel	All disciplines for new product development	Maybe only selected disciplines, others outsourced, shared
Presence in target market	Has, or will create, presence in market	May not market product, but find another channel
Scientific rigor	High; proven success record	Varies; must be high to be successful

Which Company Develops Products for Rare Disorders?

- Answer: any company which can develop a product for the amount of money justified by the potential market size of the product (ROI)
- For rare disorders, potential market size often does not support investment of time and money needed to development a product; add opportunity costs
- Collaboration among pharmaceutical company, FDA and NIH, through its SBIR grants, permits a new product development for rare disorders that otherwise would not be possible

Small company, small market

- Development of a niche product will be successful only if conducted efficiently, with clear development path, fast decisions
- Expertise of involved personnel necessary
- Prompt, frequent, efficient and effective interactions with FDA essential for success
  - can't afford time or money to do a study again
  - can't afford lengthy delays for any reason
  - must ask and answer all the correct questions

Octagen Corporation

- Small (3 person) privately-owned drug development company based in suburb of Philadelphia
- Founded 1997, based on fVIII technology licensed from Pete Lollar, MD, Emory University
- Collaborating with Ipsen, Ltd., who manufactured and marketed HYATE:C (porcine plasma-derived fVIII product); Ipsen now committed to supply OBI-1
- Objective: to develop recombinant porcine FVIII (OBI-1) as product for control of bleeds in patients with inhibitors to clotting fVIII

Need for OBI-1 in market

- Congenital hemophilia: 7 to 10% prevalence, or about 2000 patients in US, have FVIII inhibitor antibodies and require alternative Rx
- Acquired hemophilia: incidence 400 - 500 new patients/year; mortality rate still 20%
- Currently available products effective approx. 65% of time; some suggest alternate use of 2 products together
- HYATE:C no longer available
- No blood test for surrogate monitoring rVIIa or FEIBA for efficacy; can monitor FVIII level after OBI-1
- rpFVIII (OBI-1) dosing likely to be less frequent than alternative recombinant product, rFVIIa

Developing a product for *this* rare disorder

- It can be very difficult, take longer to identify and recruit fVIII inhibitor patients for clinical trials
- Patients often on home care therapy with an acceptable product, decline inconvenience of participating in a clinical trial
- However, specialty physicians usually know their long-term chronically ill patients well
  - Cong. hemophilia patients registered at HTCs
  - Acquired hemophilia patients may be referred to certain HTCs for expert management of care

Octagen Corporation structure

- In-house expertise in clinical development, including development of hemophilia products
- Octagen is collaborating with Beaufour-Ipsen
  - An established pharmaceutical company, long commitment to hemophilia with HYATE:C
  - Ipsen provides regulatory affairs function and manufacturing team, assuring quality of CMC
  - Ipsen's commitment to program shown by building entire new factory, one dedicated to recombinant products; OBI-1 is first product

NIH - SBIR Grant support

- Permits development to continue past time when ROI calculations would otherwise limit, or eventually preclude, further investment in program
- Every program seems to incur delays
  - Timelines at start had Octagen filing BLA by now, and we are now in midst of Phase II study
- Non-investment SBIR grant clearly shows NIH commitment to new products for rare “plasma” disorders, otherwise impossible

SBIR Continuation grant

- Octagen specifically extends thanks to the NHLBI for the confidence in, and support of, the OBI-1 program
- We anticipate this funding will take us far toward completing the OBI-1development
- Octagen expects this program to be one that the NIH, through its SBIR program, will point to with pride, demonstrating their support of a new product option for patients with this disorder, fVIII inhibitors, one with significant morbidity and potential mortality